Stem Cell and Gene Therapy Agency Funds Clinical Trial for a Functional Cure for HIV

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South San Francisco, CA – The use of antiretroviral drugs has turned HIV/AIDS from a fatal disease to one that can, in many cases in the US, be controlled. But these drugs are not a cure. Today the governing Board of the California Institute for Regenerative Medicine (CIRM) invested $6.85 million in a therapy that aims to cure the disease.

This is the 82nd clinical trial funded by CIRM.

There are approximately 38 million people worldwide living with HIV/AIDS. And each year there are an estimated 1.5 million new cases. The vast majority of those living with HIV do not have access to the life-saving antiretroviral medications that can keep the virus under control. People who do have access to the medications face long-term complications from them including heart disease, bone, liver and kidney problems, and changes in metabolism.

The antiretroviral medications are effective at reducing the viral load in people with HIV, but they don't eliminate it. That's because the virus that causes AIDS can integrate its DNA into long-living cells in the body and remain dormant. When people stop taking their medications the virus is able to rekindle and spread throughout the body.

Dr. William Kennedy and the team at Excision Bio Therapeutics have developed a therapeutic candidate called EBT-101. This is the first clinical study using the CRISPR-based platform for genome editing and excision of the latent form of HIV-1, the most common form of the virus that causes AIDS in the US and Europe. The goal is to eliminate or sufficiently reduce the hidden reservoirs of virus in the body to the point where the individual is effectively cured.

"To date only a handful of people have been cured of HIV/AIDS, so this proposal of using gene editing to eliminate the virus could be transformative," says Maria T. Millan, MD, President and CEO of CIRM. "In California alone there are almost 140,000 people living with HIV. HIV infection continues to disproportionately impact marginalized populations, many of whom are unable to access the medications that keep the virus under control. A functional cure for HIV would have an enormous impact on these communities, and others around the world."

The Excision Bio Therapeutics team also scored high on their plan for Diversity, Equity and Inclusion. Reviewers praised them for adding on a partnering organization to provide commitments to serve underserved populations, and to engaging a community advisory board to help guide their patient recruitment.

The CIRM Board also approved $15.6 million in funding for 11 awards under its DISC-0 program. The goal of the program is to:
• Broadly re-initiate funding of basic stem/progenitor ("stem") cell science and genetic research.
• Support rigorous studies addressing critical basic knowledge gaps or bottlenecks in regenerative medicine research.
• Advance the development of stem cell-based tools for innovation.

The awards are for three years and up to $1 million in direct costs and they will be offered twice a year.

The successful applicants are:

APPLICATION	TITLE	P.I/INSTITUTION	AMOUNT
DISC0-13901	Drug Discovery for Dilated Cardiomyopathy using Patient-Derived Human iPSCs	Syed Mukhtar Ahmed — Greenstone Biosciences	$1,350,000

DISC0-13757	Drivers of trophoblast stem/progenitor cell identity in human placenta	Francesca Soncin — University of California, San Diego	$993,881
DISC0-13801	Control of OCT4 abundance and function in human stem cells	Michael P. Rape — University of California, Berkeley	$1,415,301
DISC0-13914	Developing a Human Model of Sporadic ALS Using Machine Learning and Robotic Microscopy	Julia Kaye — The J. David Gladstone Institutes	$1,406,622
DISC0-13765	Engineering AAV capsids for transduction of neural and muscle stem cells	Melissa Jan Spencer — University of California, Los Angeles	$999,999
DISC0-13788	Modulation of human alveolar stem cells to promote lung regeneration and avoid pulmonary fibrosis	Harold A. Chapman — University of California, San Francisco	$1,626,001
DISC0-13875	Developing a microglia replacement therapy	Marius Wernig — Stanford University	$1,577,979
DISC0-13750	Generation of cortical organoids with tunable areal identities by spatial engineering of morphogens	Momoko Watanabe — University of California, Irvine	$1,497,032
DISC0-13816	Towards a trophectoderm stem-cell model representing human blastocysts of the highest implantation potential	Heidi Cook-Andersen — University of California, San Diego	$1,584,000

DISC0-13806	Development of universal off-the-shelf iPSC derived dendritic cells for use as patient specific anti-tumor vaccine	Robert Blelloch — University of California, San Francisco	$1,625,998
DISC0-13808	Development of a stem-cell based approach to interpret global effects of genetic variants contributing to neurodevelopmental disease risk	Alexis Komor — University of California, San Diego	$1,518,982

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $5.5 billion in funding and more than 150 active stem cell programs in our portfolio, CIRM is one of the world's largest institutions dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

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